Stem Cell Agency Joins Consortium Developing Gene Therapies for Rare Diseases

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South San Francisco, CA – The California Institute for Regenerative Medicine (CIRM) has signed a Memorandum of Understanding (MOU) with the Foundation for the National Institutes of Health (FNIH) to join the Bespoke Gene Therapy Consortium (BGTC), a public-private partnership, managed by FNIH, that brings together the National Institutes of Health (NIH), the U.S. Food and Drug Administration (FDA), and multiple public and private sector organizations to streamline the development and delivery of gene therapies for rare diseases.

It is estimated that there are between 25-30 million Americans living with around 7,000 rare diseases, some of these diseases are ultra-rare affecting only a few hundred, or even a few dozen people. Many of these diseases are linked to a mutation or defect in a single gene, which can be treated by highly customizable, "bespoke" gene therapy approaches.

The newly formed BGTC believes that the feasibility, efficiency, and acceleration of the development process for rare diseases will benefit from a standardized and regulatory-supported manufacturing, preclinical and clinical blueprint. That's why the BGTC aims to create a template that can be widely used to develop bespoke gene therapies quickly, more efficiently and less expensively for a wide array of intractable rare diseases.

"At CIRM we have funded several projects using gene therapy to help treat, and even cure, people with rare diseases such as severe combined immunodeficiency," says Dr. Maria T. Millan, the President and CEO of CIRM. "But even an agency with our resources can only do so much. This agreement with the Bespoke Gene Therapy Consortium will enable us to be part of a bigger partnership, one that can advance the field, overcome obstacles and lead to breakthroughs for many rare diseases."

The consortium will focus on using an adeno-associated virus (AAV) as a delivery vehicle for normal copies of genes to the right tissues and organs in the body, replacing or correcting the mutation that caused the disease. The same AAV gene delivery technology is used in commercial gene therapies for Leber congenital amaurosis (LCA) or retinitis pigmentosa (RP), caused by mutations in both copies of the RPE65 gene, and spinal muscular atrophy, and is also utilized in multiple gene therapy clinical trials for other rare and prevalent diseases. The BGTC will collaboratively drive several AAV gene therapy candidates for rare diseases from preclinical research through clinical trials and will leverage this collective manufacturing and regulatory experience to build the template for others to emulate.

Under this MOU, CIRM will identify specific rare disease gene therapy research programs in California that are eligible to be part of the AMP BGTC. CIRM funding can then support the IND-enabling research, manufacturing and clinical trial activities of these programs.

"This knowledge network/consortium model fits in perfectly with our mission of accelerating transformative regenerative medicine treatments to a diverse California and world," says Dr. Millan. "It is impossible for small, often isolated, groups of patients around the world to fund research that will help them. But pooling our resources, our skills and knowledge with the consortium means the work we support here may ultimately benefit people everywhere."

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is one of the world's largest institutions dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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